FOR IMMEDIATE RELEASE

MiNA Therapeutics Announces Findings From MTL-CEBPA Clinical Trial in Patients with Advanced Liver Cancer at International Liver Cancer Association Conference

--Oral presentation by trial's Chief Investigator describes complete tumour responses reported in patients off-study when subsequently administered standard of care--

--MiNA plans to amend current trial protocol to clinically evaluate MTL-CEBPA in combination with current standard of care for advanced liver cancer--

London, United Kingdom, September 19, 2018 – MiNA Therapeutics, the pioneer in RNA activation therapeutics, provided yesterday an update from the ongoing Phase I study of small activating RNA (saRNA) candidate MTL-CEBPA in advanced liver cancer patients. The Chief Investigator of the trial reported observations of tumour responses in three patients when administered approved liver cancer therapies subsequent to treatment with MTL-CEBPA. These responses corroborate emerging pre-clinical research on the potential for MTL-CEBPA to enhance the benefit of other cancer therapies and to modulate the tumour immune microenvironment. The update from the MiNA clinical trial was presented at the 12th Annual Conference of the International Liver Cancer Association in an oral presentation titled "First-in-Human, First-in-Class Phase I Study of MTL-CEBPA, a Small Activating RNA (saRNA) Targeting the Transcription Factor C/EBP-α in Patients with Advanced Liver Cancer" in the Novel Targets and Markers session held on Sunday, September 16, 2018.

"Although these instances are anecdotal, complete responses of tumours are a rarity in primary liver cancer," said Dr. Debashis Sarker, the Chief Investigator of the study and Principal Investigator at the National Institute for Health Research Clinical Research Facility at Guy's and St Thomas' and King's College London. "Observing two patients responding in this manner to approved cancer therapies subsequent to treatment with MTL-CEBPA is very encouraging. I am pleased that MiNA is seeking to modify its ongoing trial to include investigations on the combination in additional patients and I look forward to the opportunity to further evaluate MTL-CEBPA."

In three patients investigators initiated off-study treatment with tyrosine kinase inhibitors, 0 – 3 months after completion of on-study treatment with MTL-CEBPA. Two patients administered with sorafenib experienced confirmed complete tumour responses together with marked decreases in alpha-fetoprotein tumour marker. One of these two patients also experienced resolution of both lung and peritoneal metastases. One patient administered with lenvatinib experienced a partial tumour response. In a published Phase III study of sorafenib as a single agent, complete responses were observed in 0% of patients and partial responses were observed in 2% of patients[1]. In a published Phase III study of lenvatinib as a

[1] Llovet et al. Sorafenib in Advanced Hepatocellular Carcinoma. N Engl J Med 2008;359:378-90

single agent, complete responses were observed in 0% of patients and partial responses were observed in 18% of patients based on RECIST 1.1 criteria[2].

"These exciting observations by study investigators together with an emerging understanding of the role of CEBPA in the tumour immune microenvironment present the opportunity to evaluate a novel regimen with disease modifying potential," said Robert Habib, CEO of MiNA Therapeutics. "Having characterised in patients the safety, tolerability and saRNA proof of mechanism in a single agent setting, MTL-CEBPA is well positioned for further investigation in combination with other cancer therapies. We are in active discussions with the regulatory authorities to amend our ongoing Phase I trial to include further studies of MTL-CEBPA in combination."

The potential for MTL-CEBPA to enhance the benefits of other cancer therapies is supported by emerging pre-clinical research. In a chemically induced model of cirrhotic hepatocellular carcinoma in rats, treatment of MTL-CEBPA for one week followed by sorafenib for one week demonstrated a significant improvement in anti-tumour activity compared to either two weeks of sorafenib alone or two weeks of MTL-CEBPA alone. Durable activity of MTL-CEBPA for several weeks after treatment was previously demonstrated in pre-clinical models of liver disease[3].

In 2017 the National Cancer Institute reported pre-clinical studies demonstrating that loss of function of C/EBP-α resulted in an increase in Myeloid Derived Suppressor Cells (MDSCs) in the tumour immune microenvironment resulting in augmented tumour growth in mouse models of cancer[4]. MDSCs have been identified as key players in promoting a range of diseases, including in cancer where MDSCs may provide tumours resistance to cancer therapies.

The Phase I clinical trial of MTL-CEBPA is ongoing at multiple sites in the United Kingdom and Asia. Enrolment has been completed evaluating MTL-CEBPA as a single agent. Enrolment is expected to begin in Q4 2018 evaluating MTL-CEBPA in combination with sorafenib. For more information, please contact us at outreach@minatx.com.

About MTL-CEBPA

MTL-CEBPA consists of a double stranded RNA formulated into a SMARTICLES® liposomal nanoparticle and is designed to activate the CEBPA gene. By restoring CEBPA expression to normal levels, MTL-CEBPA has been demonstrated to attenuate or reverse liver disease in a range of pre-clinical studies including models of liver cancer, liver cirrhosis, non-alcoholic fatty liver disease (NAFLD) and non-alcoholic steatohepatitis (NASH). MTL-CEBPA is currently under evaluation in OUTREACH, a multi centre first-in-human Phase I clinical study in

[2] Kudo et al. Lenvatinib versus sorafenib in first-line treatment of patients with unresectable hepatocellular carcinoma: a randomised phase 3 non-inferiority trial. Lancet 2018 391; 10126: 1163-1173

[3] Reebye et al. Gene activation of CEBPA using saRNA: preclinical studies of the first in human saRNA drug candidate for liver cancer. Oncogene 2018 37: 3216-3228

[4] Mackert et al. Dual negative roles of C/EBPα in the expansion and pro-tumor functions of MDSCs. Scientific Reports 2017; 7: 14048

patients with severe liver cancer. In preliminary results from the OUTREACH study, MTL-CEBPA was generally well tolerated in patients with both healthy and impaired liver function was found to mediate RNAa activity in white blood cells. To learn more about the OUTREACH clinical study, please visit our listing at clinicaltrials.gov

About MiNA Therapeutics
Harnessing an innate mechanism of gene activation, MiNA Therapeutics' platform enables the development of new medicines that restore normal function to patients' cells. We are applying our technology and clinical know-how to transform the therapy landscape of severe liver and other diseases. www.minatx.com

About the NIHR Clinical Research Facility at Guy's and St Thomas' NHS Foundation Trust
Our Clinical Research Facility (CRF) provides world-leading facilities and expertise to support the NHS, universities and industry in conducting experimental medicine studies.

It is unique in that it:

- has achieved MHRA Phase I accreditation (UK Medicines and Healthcare Regulatory Authority) for our Guy's Hospital unit – the gold standard in the UK for delivering Phase I clinical trials
- is the only UK CRF with embedded Advanced Therapies Manufacturing (GMP) and Immune Monitoring platforms
- has four specialist units: a Phase I Unit at Guy's Hospital, an Imaging Unit and Paediatric Children's Unit within Evelina London Children's Hospital and a Cardiometabolic Unit at St Thomas' Hospital.

With management and infrastructure of the CRF embedded within the NIHR Biomedical Research Centre (BRC) at Guy's and St Thomas' and King's College London, the CRF is able to efficiently take clinical studies through the experimental medicine pipeline to new and improved treatments for patients.
www.guysandstthomasbrc.nihr.ac.uk

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